

CIRM Funded Clinical Trials

Phase 2 Study of Hematopoietic Stem Cell Gene Transfer Inducing Fetal Hemoglobin in Sickle Cell Disease

Disease Area:	Sickle Cell Disease
Investigator:	David Williams
Institution:	Boston Children's Hospital
CIRM Grant:	CLIN2SCD-12031 (Pre-Active)
Award Value:	\$8,333,581
Trial Sponsor:	Boston Children's Hospital
Trial Stage:	Phase 2
Trial Status:	Launching
Targeted Enrollment:	N/A



David Williams

Details:

Boston Children's Hospital is conducting a gene therapy clinical trial for sickle cell disease (SCD). This project is part of an agreement between CIRM and the National Heart, Lung, and Blood Institute (NHLBI), part of the National Institutes of Health, to co-fund cell and gene therapy programs under the NHLBI's "Cure Sickle Cell" Initiative. The goal of this agreement is to markedly accelerate clinical development of cell and gene therapies to cure SCD.

SCD is an inherited disease caused by a single gene mutation resulting in abnormal hemoglobin, which causes red blood cells to 'sickle' in shape. Sickling of red blood cells clogs blood vessels and leads to progressive organ damage, pain crises, reduced quality of life, and early death.

The team will take a patient's own blood stem cells and insert a novel engineered gene to silence abnormal hemoglobin and induce normal fetal hemoglobin expression. The modified blood stem cells will then be reintroduced back into the patient. The goal of this therapy is to aid in the production of normal shaped red blood cells, thereby reducing the severity of the disease.

Design:

This is a Phase II study

Goal:

Safety and efficacy.